



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0229]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (the FD&C Act), as amended by the Food and Drug Administration Safety and Innovation Act (FDASIA), authorizes FDA to award priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. FDA has determined that EXONDYS 51 (eteplirsen), manufactured by Sarepta Therapeutics, meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Larry Bauer, Rare Diseases Program, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-796-4842, FAX: 301-796-9858, email:

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SUPPLEMENTARY INFORMATION:

FDA is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), which was added by FDASIA, FDA will award priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. FDA has determined that

EXONDYS 51 (eteplirsen), manufactured by Sarepta Therapeutics, meets the criteria for a priority review voucher. EXONDYS 51 (eteplirsen) is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm>. For further information about EXONDYS 51 (eteplirsen) go to the “Drugs@FDA” Web site at <http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm>.

Dated: October 6, 2016.

Leslie Kux,

Associate Commissioner for Policy.

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